Celiac Disease
FOUNDATION
celiac.org

Celiac Disease
Patient Advocacy Program

Goal

Participants will improve their medical, scientific, and advocacy skills so they can better the design, implementation, and dissemination of celiac disease and non-celiac wheat sensitivity research.

Medical and Scientific Learning Objectives

Participants will develop a working knowledge of:

- Current practices in the screening of celiac disease
- Current practices in the diagnosing of celiac disease
- The underlying genetics that cause celiac disease
- The pathway to drug development
- Types and phases of clinical trials
- Label reading to prevent gluten exposure

Advocacy Skills Learning Objectives

Participants will develop a working knowledge of:

- The purpose and unique features of patient-centered outcomes research (PCOR)
- The roles for patients and stakeholders as partners in research
- The purpose of the Celiac Disease Foundation Patient Advocacy Program

Agenda

Session One: Patient-Centered Outcomes Research

Session Two: Celiac Disease 101

Session Three: Genetics

Session Four: Clinical Trials

Session Five: Nutrition Labels

Session Six: Patient-Centered Research Questions

Session One

Patient-Centered Outcomes Research

Session One Objectives

You will learn:

- The purpose, goals, and unique aspects of Patient-Centered Outcomes Research (PCOR)
- The purpose and goals of the Patient-Centered Outcomes Research Institute (PCORI)
- The purpose and goals of the CDF Patient Advocacy Program

Acronyms

We'll try to avoid them, but…

CDF = Celiac Disease Foundation

PCOR = Patient-Centered Outcomes Research

PCORI = Patient-Centered Outcomes Research Institute

CER = Comparative Effectiveness Research

iCureCeliac = CDF's patient registry

What is Patient-Centered Outcomes Research?

Comparative Effectiveness Research (CER)

The *direct comparison* of existing healthcare interventions to determine which work best for which patients and which pose the greatest benefits and harms

What works best?

For which patients?

Under what circumstances?

Patient-Centered Outcomes Research

Patient-Centered Outcomes Research is a type of **Comparative Effectiveness Research** that answers patient-centered questions, such as:

- Given my personal characteristics, conditions, and preferences, what should I expect will happen to me?
- What are my options, and what are the potential benefits and harms of these options?
- How can clinicians and the care delivery systems work to help me make the best decisions about my health and healthcare?
- What can I do to improve the outcomes that are most important to me?

Different Types of Trials

- Efficacy Trials
  - Does it work (produce the intended effect) in ideal circumstances?

- Effectiveness Trials or Pragmatic Trials
  - Does it work in the real world?

A Few Terms

Patient Engagement

Inclusion of patients in the research process, from topic selection through study design and conduct to dissemination of findings.

IS NOT THE SAME AS

Patient-Centered

Addresses questions that patients and their families care about in clinical settings.

A Few More Terms

Patient-Reported Outcomes

Any report of the status of a patient's health condition that comes directly from the patient, without interpretation of the patient's response by a clinician or anyone else.

IS NOT THE SAME AS

Patient-Centered Outcomes

Research that is specifically designed to meet the most important needs of patients. Relevant data can come directly from patients, or from anywhere else.

Terms you may also hear…

Patient-Focused Research

Person-Focused Research

Community-Based Research

Participatory Research

Collaborative Research

Patient-Centered Outcomes Research Institute (PCORI)

Research Done Differently

Patient-Centered Outcomes Research Institute (PCORI)

- Created by Congress under the Affordable Care Act to figure out which medical treatments work best
- Criteria for evaluation includes patient engagement and patient-centeredness
- Applications are reviewed by scientists, patients, and other stakeholders
- Began funding research in December 2012
- Expects to spend $3.5 billion by the end of the decade

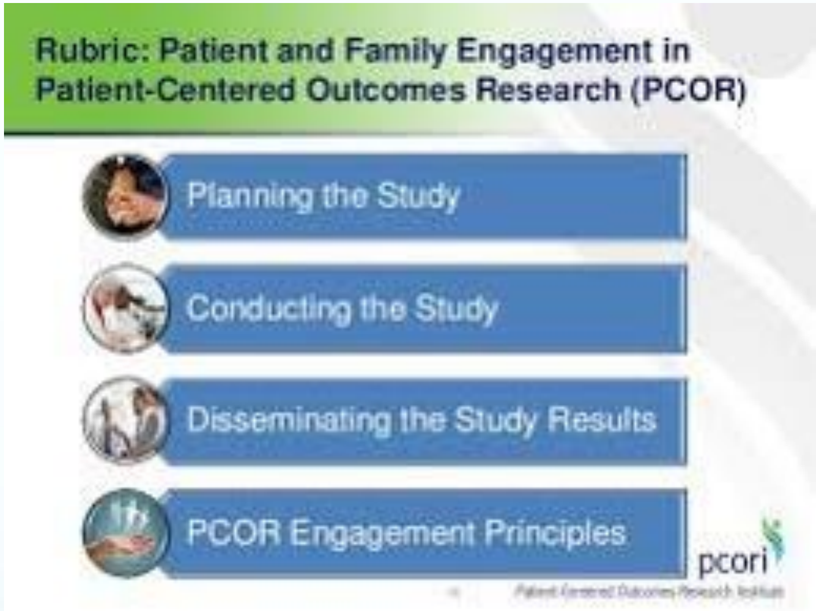
Patient-Centered Outcomes Research Institute (PCORI)

Patient-centered is not enough—

Good science is not enough—

It must be BOTH!

Patient-Centered Outcomes Research Institute (PCORI)



Rubric: Patient and Family Engagement in Patient-Centered Outcomes Research (PCOR)

- Planning the Study
- Conducting the Study
- Disseminating the Study Results
- PCOR Engagement Principles

Patients Can Participate in Planning Research by ...

Developing the research question and relevant outcomes to be studied.

Why? *To ensure that the project and its results will be useful and important to patient and stakeholder communities.*

Defining the characteristics of study participants.

Why? *To minimize the risk that certain patients will be included or excluded due to criteria that are not relevant.*

Drafting or revising study materials and protocols.

Why? *To promote/increase retention of study participants.*

Patients Can Participate in Conducting Research by ...

Drafting or revising study materials and protocols.

Why? *To ensure feasibility for clinicians and patient participants.*

Participating in recruitment of study participants.

Why? *To increase and sustain recruitment, and to ensure viability of the study.*

Participating in data collection and data analysis.

Why? *To lend unique and varied perspectives on interpretation of the data.*

Patients Can Participate in Conducting Research by ...

Serving as a patient representative on a data safety monitoring board.

Why? *To make the board composition more robust and patient-centered.*

Participating in the evaluation of patient and stakeholder engagement.

Why? *To ensure authenticity and value of engagement.*

Patients Can Participate in Disseminating Research by ...

Identifying partner organizations for dissemination.

Why? *To ensure meaningful and direct connections with end-users.*

Planning dissemination in the context of shaping study design and protocol.

Why? *To ensure dissemination is incorporated into the research from the very beginning.*

Patients Can Participate in Disseminating Research by ...

Authoring manuscripts and presenting study findings.

Why? *To offer the patient perspective and to reach new and different audiences.*

Identifying opportunities to share information about the study, even as it is in progress.

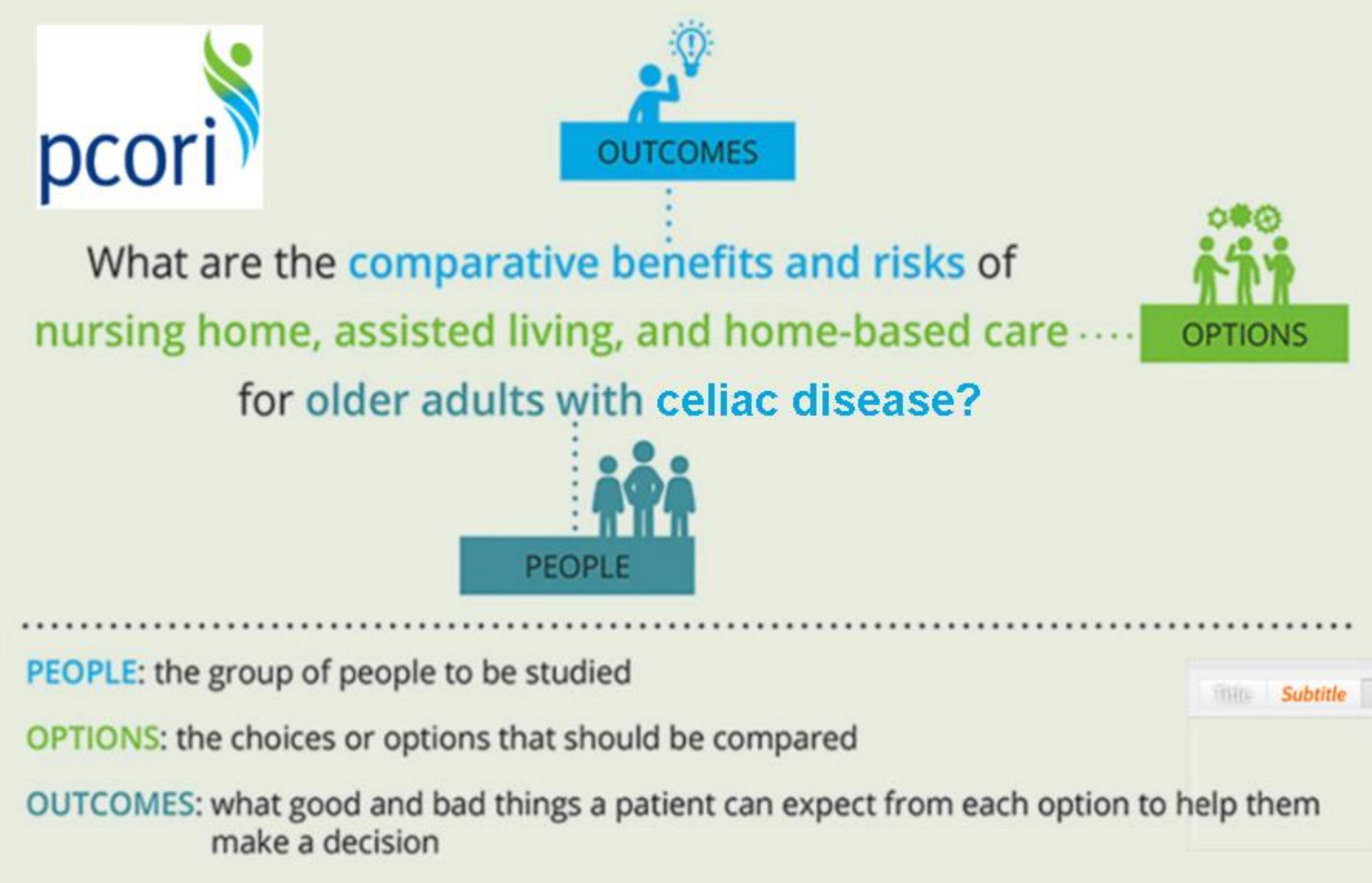
Why? *To move away from traditional models of dissemination and think more creatively about how to get information into the hands of those who need it.*

Translating Our Concerns and Questions into Research

The People Who are the people who should be studied? This is the population of interest.

The Options How can people make informed choices between options? These are the factors that people will consider when making a decision between/among options.

The Outcome What options should be compared? These are the decisions the research is intended to inform.



What are the comparative benefits and risks of nursing home, assisted living, and home-based care for older adults with celiac disease?

PEOPLE: the group of people to be studied

OPTIONS: the choices or options that should be compared

OUTCOMES: what good and bad things a patient can expect from each option to help them make a decision

CDF Patient Advocacy Program

Purpose

To develop a nationwide network of patient advocates who can help to design, implement, and disseminate results of patient-centered outcomes research that are important to adults and children with celiac disease and caregivers.

CDF Patient Advocacy Program

Goal

Establish a national network of 200 celiac disease patient advocates who are ready, willing, and able to collaborate with the research community by the end of 2017.

Review: Session One Objectives

You will learn:

- The purpose, goals, and unique aspects of Patient-Centered Outcomes Research (PCOR)
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- The purpose and goals of the Celiac Disease Patient Advocacy Program

Complete the Understanding Check